



US009511074B2

(12) **United States Patent**
Perlmutter et al.

(10) **Patent No.:** **US 9,511,074 B2**
(45) **Date of Patent:** **Dec. 6, 2016**

(54) **METHODS OF TREATING DISORDERS
ASSOCIATED WITH PROTEIN
POLYMERIZATION**

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(*) Notice: Subject to any disclaimer, the term of this
patent is extended or adjusted under 35
U.S.C. 154(b) by 0 days.

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(22) Filed: **Nov. 6, 2014**

(65) **Prior Publication Data**

US 2015/0065488 A1 Mar. 5, 2015

Related U.S. Application Data

(63) Continuation of application No. 13/362,606, filed on
Jan. 31, 2012, now Pat. No. 8,906,905, which is a
continuation-in-part of application No.
PCT/US2010/044243, filed on Aug. 3, 2010.

(60) Provisional application No. 61/230,921, filed on Aug.
3, 2009.

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(51) **Int. Cl.**
A61K 31/55 (2006.01)
A61K 31/155 (2006.01)

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(52) **U.S. Cl.**
CPC **A61K 31/55** (2013.01); **A61K 31/155**
(2013.01)

(57) **ABSTRACT**

(58) **Field of Classification Search**
CPC A61K 31/55
See application file for complete search history.

The present invention relates to methods of treatment of
clinical disorders associated with protein polymerization
comprising administering, to a subject, an effective amount
of carbamazepine, oxcarbazepine or another carbam-
azepine-like compound. It is based, at least in part, on the
discovery that, in cells having a genetic defect in α 1-antitrypsin,
carbamazepine was able to decrease levels of the
mutant protein. Furthermore, carbamazepine reduced the
hepatic load of mutant α 1-antitrypsin and the toxic effect of
that mutant protein accumulation, hepatic fibrosis, in vivo
using a mouse model of the disease. As patients having this
defect in α 1-antitrypsin exhibit toxic accumulations of the
protein, treatment according to the invention may be used to
ameliorate symptoms and signs of disease.

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4 Claims, 14 Drawing Sheets